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103D CONGRESS
2D SESSION

H. R. 4160

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

IN THE HOUSE OF REPRESENTATIVES

MARCH 24, 1994

Mr. WAXMAN (for himself and Mr. STUDDS) introduced the following bill; which was referred to the Committee on Energy and Commerce

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A BILL

To amend the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act, and the Orphan Drug Act to revise the provisions of such Acts relating to orphan drugs.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE AND REFERENCE.

(a) SHORT TITLE.—This Act may be cited as the ''Orphan Drug Act Amendments of 1994 ''.

(b) REFERENCE.—Whenever in this Act (other than sections 5 and 6) an amendment or repeal is expressed in terms of an amendment to, or repeal of, a section or

other provision, the reference shall be considered to be made to a section or other provision of the Federal Food, Drug, and Cosmetic Act.

SEC. 2. PERIOD OF EXCLUSIVITY.

(a) INITIAL PERIOD.—Subsection (a) of section 527 (21 U.S.C. 360cc) is amended—

(1) by inserting ''(1)'' after ''(a)'',

(2) by redesignating paragraphs (1), (2), and (3) as subparagraphs (A), (B), and (C), respectively,

(3) by striking ''seven years'' and inserting ''4 years'', and

(4) by striking ''505(c)(2)'' and inserting ''505(c)(1)(B)''.

(b) ADDITIONAL PERIOD.—Subsection (a) of such section 527 (21 U.S.C. 360cc) (as amended by subsection (a)) is amended by adding at the end the following:

''(2) The holder of the approved application, certification, or license of a drug to which the 4-year period of exclusivity applies under paragraph (1) may, after the expiration of 3½ years of such period but not later than 90 days before the expiration of such period, apply to the Secretary for a 3-year extension of such period. Such an application shall contain such information as the Secretary determines is necessary to evaluate such application.

''(3) The Secretary shall approve an application submitted under paragraph (2) if the applicant—

''(A) demonstrates that the drug has a limited commercial potential as determined under regulations of the Secretary, taking into account sales information respecting such drug and any other factor identified by the Secretary in such regulations which is relevant to the commercial potential of such drug, and

''(B) makes such demonstration on the basis of the regulations of the Secretary referred to in subparagraph (A) which were in effect—

''(i) on the date—

''(I) such drug received its designation under section 526(a), or

''(II) such applicant applied for an exemption for such drug under section 505(i) or 507(d),

whichever first occurs, or

''(ii) if the date under clause (i) occurred before the date such regulations were in effect, or the date such regulations were in effect.''.

(c) CONFORMING AMENDMENT.—Section 527(b) (21 U.S.C. 360cc(b)) is amended—

(1) by striking ''during the seven-year period beginning on the date of the application approval'' and inserting ''during the applicable period of exclusivity under subsection (a)'', and

(2) by striking ''such seven year period'' and inserting ''the applicable period of exclusivity under subsection (a)''.

(d) EFFECTIVE DATE.—The amendments made by subsections (a) and (b) shall not apply to a drug—

(1) for which an application under section 505 or 507 of the Federal Food, Drug, and Cosmetic Act or section 351 of the Public Health Service Act was submitted before March 1, 1994, or

(2) for which an exemption under section 505(i) or 507(d) of the Federal Food, Drug, and Cosmetic Act was in effect before March 1, 1994, for which human clinical trials were actively being conducted before such date, and for which an application for designation under section 526 of such Act was submitted before the date of enactment of the Orphan Drug Act Amendments of 1994.

The 7 year period of exclusivity provided by section 527(a) of the Federal Food, Drug, and Cosmetic Act before the date of the enactment of this Act shall, after such date, apply to a drug described in paragraph (1) or (2).

(e) REGULATIONS.—The Secretary shall issue final regulations to implement paragraphs (2) and (3) of section 527(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360cc) (as amended by subsection (b)) not later than 6 months after the date of the enactment of this Act.

SEC. 3. DESIGNATIONS.

(a) IN GENERAL.—Section 526(a)(2) (21 U.S.C. 360bb(a)(2)) is amended to read as follows:

''(2) For purposes of paragraph (1), the term 'rare disease or condition' means any disease or condition which—

''(A) affects fewer than 200,000 persons in the United States determined on the basis of—

''(i) the facts and circumstances as of the date the request for designation of the drug under this subsection is made, and

''(ii) projections as to the number of persons who will be affected by the disease or condition on a date which is 3 years from date such request was made, or

''(B) affects more than 200,000 persons in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such dis-

ease or condition will be recovered from sales in the United States of such drug.''.

(b) EXCLUSIVITY.—Section 527(b) (21 U.S.C. 360cc(b)) is amended—

(1) in paragraph (1), by striking ''The'' and inserting ''the'' and by striking ''or'' at the end of such paragraph,

(2) by striking the period at the end of paragraph (2) and inserting ''; or'', and

(3) by adding at the end the following:

''(3) a drug has been designated under section 526 for a rare disease or condition described in section 526(a)(2)(A) and if after such designation it is determined that—

''(A) such disease or condition affects more than 200,000 persons in the United States, and

''(B) such drug does not meet the requirement of section 526(a)(2)(B).''.

SEC. 4. SIMULTANEOUS DEVELOPMENT.

(a) IN GENERAL.—Section 527(b) (21 U.S.C. 360cc(b)), as amended by section 3(b), is amended by inserting ''(1)'' after ''(b)'', by redesignating paragraphs (1), (2), and (3) as subparagraphs (A), (B), and (C), respectively, by striking ''for a person who is not'' and by

inserting ''for an applicant who is not'', and by adding at the end the following:

''(D) the Secretary finds, after providing the holder, such applicant, and any other interested person an opportunity to present their views, that the drugs of the holder and such applicant were developed simultaneously.

The Secretary shall make a decision on a request for a finding under subparagraph (D) not later than 60 days after the filing of the request.

''(2) For purposes of paragraph (1)(D), drugs of a holder and an applicant shall be considered to be developed simultaneously only if—

''(A) the applicant requested that its drug be designated under section 526 no later than 6 months after publication of the designation under section 526(c) of the holder's drug,

''(B) the applicant initiated the human clinical trials that the applicant relied on in its application for such approval, certification, or license not more than 12 months after the date the holder initiated the human clinical trials that the holder relied on in its application for such approval, certification, or license, and

''(C) the applicant submitted such application, including the reports of the clinical and animal studies necessary for approval, certification, or licensing, not more than 12 months after the holder submitted its application, including such reports, for such action.

''(3) Paragraph (1)(D) does not apply to a drug—

''(A) for which an application under section 505 or 507 or section 351 of the Public Health Service Act was submitted before March 1, 1994, or

''(B) for which an exemption under section 505(i) or 507(d) was in effect before March 1, 1994, for which human clinical trials were actively being conducted before such date, and for which an application for designation under section 526 was submitted before the date of enactment of the Orphan Drug Act Amendments of 1994.''.

(b) PUBLICATION.—Section 526(c) (21 U.S.C. 360bb(c) is amended—

(1) by inserting ''for a rare disease or condition'' after ''(a)'', and

(2) by striking out ''shall be made available to the public'' and inserting in lieu thereof ''shall be promptly published in the Federal Register and otherwise made available to the public in a manner de-

signed to notify persons who have such disease or condition''.

SEC. 5. OFFICE FOR ORPHAN DISEASES AND CONDITIONS.

Section 227 of the Public Health Service Act (42 U.S.C. 236) is amended—

(1) by amending subsection (a) to read as follows:

''(a) There is established in the Department of Health and Human Services an Office for Orphan Diseases and Conditions. Such Office shall be established at a level within the Department with sufficient authority to assure full implementation of the functions and responsibilities established by this section.'',

(2) by striking ''Board'' each place the term appears and inserting ''Office'',

(3) by striking ''drugs and devices'' in subsection (b) and inserting ''drugs, devices, and medical foods'',

(4) by inserting ''of chapter V'' after ''subchapter B'' in subsection (c)(1)(A),

(5) by adding at the end the following new subsection:

''(f)(1) There is established in the Office an advisory committee to advise the Office in carrying out the functions of the Office under this section.

''(2) The advisory committee shall be comprised of 11 members appointed by the Secretary, in consultation with the Office and the Commissioner of the Food and Drug Administration, from persons knowledgeable about rare diseases and conditions, including—

''(A) 5 representatives of organizations of persons with rare diseases or conditions;

''(B) 3 research scientists; and

''(C) 3 representatives of health-related companies.

''(3) The Secretary shall also appoint, as liaisons to the advisory committee, individuals from the Food and Drug Administration, the National Institutes of Health, and other appropriate Federal agencies.

''(4) Vacancies occurring in the membership of the advisory committee shall be filled in the same manner as the original appointment for the position being vacated. Vacancies shall not affect the power of the remaining members to execute the duties of the advisory committee.

''(5) Members of the advisory committee, and liaisons to the advisory committee, shall not be compensated, but shall receive travel expenses, including per diem in lieu of subsistence, at rates authorized for employees of agencies under subchapter 1 of chapter 57 of title 5, United States Code, for each day the member or liaison is en-

gaged in the performance of duties away from the home or regular place of business of the member or liaison.

''(6) Notwithstanding section 1342 of title 31, United States Code, the advisory committee may accept the voluntary services provided by a member of the advisory committee or a liaison to the advisory committee.'', and

(6) by amending the section heading to read as follows: ''OFFICE FOR ORPHAN DISEASES AND CONDITIONS''.

SEC. 6. AUTHORIZATION FOR ORPHAN DRUG ACT.

Section 5(c) of the Orphan Drug Act (21 U.S.C. 360ee(c)) is amended by striking ''$10,000,000'' and all that follows and inserting ''$20,000,000 for fiscal year 1995, $25,000,000 for fiscal year 1996, and $30,000,000 for fiscal year 1997.''.

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